

What is claimed is:

1. A method for treating a neurodegenerative disease in a subject comprising:
identifying a target site in the central nervous system that requires modification;
delivering a vector comprising a nucleotide sequence encoding a glutamic acid
5 decarboxylase (GAD) to the target site in the central nervous system; and
expressing the GAD in the target site in an amount effective to treat or reduce the
neurodegenerative disease.
2. The method of claim 1, wherein the vector is a viral vector.
- 10 3. The method of claim 2, wherein the a viral vector is selected from the
group consisting of adenovirus vectors, herpes virus vectors, parvovirus vectors, and
lentivirus vectors.
4. The method of claim 2, wherein the a viral vector is an adeno-associated viral
15 vector.
5. The method of claim 1, wherein the vector is a non-viral vector.
6. The method of claim 5, wherein the non-viral vector is a liposome-mediated
delivery vector.
- 20 7. The method of claim 1, wherein the vector is delivered using stereotaxic delivery.
8. The method of claim 1, wherein the target site in the central nervous system is a
region of the brain.
- 25 9. The method of claim 8, wherein the region of the brain is selected from the group
consisting of basal ganglia, subthalamic nucleus (STN), pedunculopontine nucleus (PPN),
substantia nigra (SN), thalamus, hippocampus, cortex, and combinations thereof.
10. The method of claim 8, wherein the region of brain is the subthalamic nucleus
30 (STN).

11. The method of claim 1, wherein the neurodegenerative disease is selected from the group consisting of Parkinson's disease, Alzheimer's disease, senile dementia, Amyloid Lateral Schlerosis (ALS), and epilepsy.
- 5 12. A method for treating Parkinson's disease in a subject comprising:
identifying one or more regions of the brain that require modification;
delivering a vector comprising a nucleotide sequence encoding a glutamic
acid decarboxylase (GAD) to the region of the brain; and
expressing the GAD in the region of the brain an amount effective to treat
10 or reduce Parkinson's disease.
13. The method of claim 12, wherein the vector is a viral vector.
14. The method of claim 13, wherein the a viral vector is selected form the group
consisting of adenovirus vectors, herpes virus vectors, parvovirus vectors, and lentivirus
15 vectors.
15. The method of claim 13, wherein the a viral vector is an adeno-associated viral
vector.
16. The method of claim 12, wherein the vector is a non-viral vector.
- 20 17. The method of claim 16, wherein the non-viral vector is a liposome-mediated
delivery vector.
18. The method of claim 12, wherein the region of the brain is selected from the
group consisting of basal ganglia, subthalamic nucleus (STN), pedunclopontine nucleus
(PPN), substantia nigra (SN), thalamus, hippocampus, cortex, and combinations thereof.
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19. The method of claim 12, wherein the region of brain is the subthalamic nucleus
(STN).
20. A vector for expression of GAD in cells of the central nervous system comprising:
30 a tissue specific promoter operably linked to a nucleotide sequence encoding
GAD; and
a post-transcriptional regulatory element.

21. The vector of claim 20, wherein the vector is selected from the group consisting of adenovirus vectors, herpes virus vectors, parvovirus vectors, and lentivirus vectors.

5 22. The vector of claim 21, wherein the vector is an adeno-associated vector.

23. The vector of claim 20, wherein the promoter is the neuron specific enolase (NSE) promoter.

10 24. The vector of claim 20, wherein the post-transcriptional regulatory element is the woodchuck post-transcriptional regulatory element.

25. The vector of claim 20, wherein the GAD is selected from the group consisting of GAD-65 and GAD-67.

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